CONGRESSIONAL BUDGET OFFICE
COST ESTIMATE

July 15, 2003

S. 650
Pediatric Research Equity Act of 2003

As reported by the Senate Committee on Health, Education, Labor, and Pensions on June 27, 2003

SUMMARY

S. 650 would authorize the Food and Drug Administration (FDA) to require drug manufacturers to assess the safety and effectiveness of drugs and biological products for use in children, and to establish dosing and administration protocols. The bill's authority would be retroactive to October 17, 2002, when a federal district court overturned the FDA's Pediatric Rule.

The bill would require applicants seeking approval of new drugs or biological products, new indications, new dosage forms, new dosing regiments, or new routes of administration to include in the application an assessment of the effect of the drug in pediatric patients. In addition, the Secretary of Health and Human Services could require the holder of an approved application to conduct an assessment of the effect of the drug or biological product in pediatric patients if the Secretary finds that the absence of pediatric labeling could pose a significant risk to children and the drug either is used in a substantial number of pediatric patients for the labeled indication or there is reason to believe that the drug would provide a therapeutic benefit over existing therapies for a claimed indication.

S. 650 would result in the collection and spending of additional user fees for already-approved drugs that would be required to conduct an assessment. CBO estimates that the FDA would collect and spend an additional $2 million in 2004 and $8 million over the 2004-2008 period in user fees. In addition, S. 650 would result in spending of $1 million in 2004 and $7 million over the 2004-2008 period over and above the amount collected for user fees. These costs consist of amounts required by the FDA to implement and administer the activities authorized under the bill.

CBO expects that enacting S. 650 would affect direct spending and revenues, but we cannot determine whether that effect would be to increase or reduce direct spending or revenues. On one hand, the bill would result in some drugs receiving a six-month extension of the period of market exclusivity, thereby delaying the entry of lower-priced generic drugs and increasing spending by federal health programs—including direct spending for Medicaid and the Federal Employees Health Benefits (FEHB) program—and by private health insurance plans. (Higher spending by private health insurance plans would cause a shift in compensation from taxable wages to tax-favored benefits, thereby reducing federal revenue from income taxes and payroll taxes.) On the other hand, the research on the effect of drugs in pediatric patients would likely result in fewer adverse reactions and medical errors, thereby reducing spending by federal and private health insurance programs. CBO cannot estimate whether the net effect of the bill would be to increase or reduce spending by health insurance programs.

S. 650 contains no intergovernmental mandates as defined in the Unfunded Mandates Reform Act (UMRA). State, local, and tribal governments, as administrators of the Medicaid program and as providers of health care coverage for their employees, may realize both costs and savings as a result of provisions in the bill, but those effects are expected to be small. Provisions affecting market and pediatric exclusivity would result in some added costs, but reductions in adverse reactions and medical errors as a result of additional research required by the bill would result in some savings.

The bill contains private-sector mandates on manufacturers of new and previously approved drugs by requiring them to perform studies of the safety and efficacy of those drugs on pediatric populations. CBO estimates that the direct cost of the new requirements would exceed the annual threshold specified in UMRA ($117 million in 2003, adjusted annually for inflation) in each of the first five years the mandates would be effective.

ESTIMATED COST TO THE FEDERAL GOVERNMENT

The estimated cost of S. 650 is shown in the following table. The costs of this legislation would fall within budget functions 050 (national defense), 550 (health), and 700 (veterans benefits and services).

	By Fiscal Year, in Millions of Dollars				
	2004	2005	2006	2007	2008
CHANGES IN SPENDING SUBJECT TO APPROPRIATION [a]					
Collection of FDA User Fees					
Estimated Authorization Level	-2	-2	-2	-2	0
Estimated Outlays	-2	-2	-2	-2	0
Spending of FDA User Fees					
Estimated Authorization Level	2	2	2	2	0
Estimated Outlays	2	2	2	2	0
FDA Administrative Expenses					
Estimated Authorization Level	1	1	1	1	3
Estimated Outlays	1	1	1	1	3
Net Effect on Spending by the Food and Drug Administration					
Estimated Authorization Level	1	1	1	1	3
Estimated Outlays	1	1	1	1	3

a. Enacting S. 650 also would probably affect direct spending and revenues, but CBO cannot estimate the magnitude or the sign (net cost or savings) of such changes.

BASIS OF ESTIMATE

Spending by FDA

S. 650 would authorize the FDA to require research on new and already approved drugs and biologicals to assess the safety and effectiveness for there use in children. Assuming appropriation of the necessary amounts, CBO estimates that implementing S. 650 would result in new net costs for the FDA of $1 million in 2004 and $7 million over the 2004-2008 period.

Under current law, the FDA is required to assess and collect application and other user fees from manufacturers of drugs to expedite the review of the drug application. (That requirement will expire at the end of 2007.) Therefore, applicants for approval of new drugs will submit user fees to the FDA under current law. However, S. 650 would increase the number of supplemental applications for already-approved drugs that would be required to

complete a pediatric assessment, resulting in an increase in the user fees collected by the FDA. CBO estimates that S. 650 would result in the collection and spending of user fees of $2 million in 2004 and $8 million over the 2004-2007 period.

S. 650 would permit the Secretary of Health and Human Services to defer the submission of the pediatric assessment for a drug or biological product if the product is expected to be ready for use in adults long before pediatric studies are complete, if there are significant safety concerns, or if the Secretary identifies some other appropriate reason for deferral. Similarly, the Secretary could grant full or partial waiver from the requirement to conduct a pediatric assessment if there is evidence that the necessary studies are impossible or impracticable to conduct, if the drug or biological is ineffective or unsafe for use in children, or the product does not represent a meaningful therapeutic benefit over existing products and the product is not likely to be used for children. If the Secretary grants a full or partial waiver because the product is ineffective or unsafe for use in children, the bill would require that information to be included in the labeling of the product.

Spending by Federal and Private Health Insurance Plans

Under current law, manufacturers can conduct pediatric research and apply for pediatric exclusivity. Pediatric exclusivity refers to a six-month period during which the FDA will not permit another manufacturer to market a version of a drug. CBO assumes that manufacturers of drugs with large sales volumes generally will conduct pediatric research and apply for pediatric exclusivity under current law. However, manufacturers of drugs with small sales volumes generally will not conduct pediatric research because the additional profits from six months of exclusivity will not outweigh the costs associated with conducting the research. Therefore, this bill would have a larger effect on drugs with small sales volumes, some of which would now apply for pediatric exclusivity.

If S. 650 is enacted, CBO estimates that approximately 50 studies would be required each year. Some manufacturers who would be required to submit assessments under this bill also would submit an application and be granted pediatric exclusivity. That exclusivity period would delay the entry of generic versions of the drugs and, therefore, would result in higher prices for affected drugs. CBO estimates that the higher prices would increase costs for federal programs—including direct spending for Medicaid and the FEHB program—and private health insurance plans. Higher prices for affected drugs would result in higher spending by private health insurance plans which would cause a shift in compensation from taxable wages to tax-favored benefits, thereby reducing federal revenue from income taxes and payroll taxes.

At the same time, however, the information gained from pediatric research and the improved labeling and prescribing patterns would likely result in fewer adverse reactions and medical

errors, thereby reducing spending by federal and private health insurance plans. CBO cannot estimate whether the net effect of the bill would be to increase or reduce spending by health insurance programs. Therefore, CBO cannot estimate whether the bill would increase or reduce direct spending or revenue; in either case, we expect the effect would be small.

If a required assessment is not submitted to the FDA the product associated with the required assessment would be considered misbranded and subject to relevant enforcement. However, the product would not be subject to penalties, criminal procedures, or fines. In addition, a failure to submit an assessment could not result in a product's approval or license being revoked.

ESTIMATED IMPACT ON STATE, LOCAL, AND TRIBAL GOVERNMENTS

S. 650 contains no intergovernmental mandates as defined in UMRA. State, local, and tribal governments, as administrators of the Medicaid program and as providers of health care coverage for their employees, may realize both costs and savings as a result of provisions in the bill, but those effects are expected to be small. Provisions affecting market and pediatric exclusivity would result in some added costs, but reductions in adverse reactions and medical errors as a result of additional research required by the bill would result in some savings.

ESTIMATED IMPACT ON THE PRIVATE SECTOR

The bill contains private-sector mandates as defined in UMRA on drug manufacturers. Under current law, drug manufacturers must comply with certain requirements set by the FDA before offering their products for sale on the U.S. market. S. 650 would expand those requirements both for manufacturers of new drugs (drugs for which new drug applications and supplemental new drug applications will be required) as well as for manufacturers of previously approved drugs. CBO estimates that the direct cost of the new requirements in the bill would exceed the annual threshold specified in UMRA ($117 million in 2003, adjusted annually for inflation) in each year those mandates would be effective.

Currently, the FDA has the authority to request that manufacturers who submit new drug applications provide it with information on the safety and efficacy of those new drugs in all relevant pediatric subpopulations. (If a manufacturer voluntarily agrees to perform the studies in response to a written request from the FDA, the FDA may extend by six months the period of market exclusivity provided to that manufacturer for their new drug.) Section 2 of S. 650 would give the FDA the authority to require drug manufacturers to perform similar studies. In addition, the bill also would give the FDA the authority to require manufacturers to develop appropriate formulations of the drugs for each pediatric age group.

CBO estimates that the direct cost of those provisions to manufacturers of new drugs would be about $135 million in 2004. Between April 1999 and March 2002, both the (voluntary) pediatric exclusivity program and a provision similar to the one in S. 650 allowing the FDA to require pediatric safety and efficacy studies were in effect. Based on the number of new drug applications for which studies were required during that period, CBO estimates studies for approximately 50 new drugs would be required under the bill each year. Further, based on a survey sponsored by the Tufts Center for the Study of Drug Development, CBO estimates that the average cost of such pediatric studies would be approximately $2.5 million per new drug. (In addition, CBO estimates that the direct cost of requiring manufacturers to develop appropriate formulations of new drugs would be approximately $10 million annually.)

Section 2 also would give the FDA the authority to require manufacturers of previously approved drugs to undertake studies of the safety and efficacy of those drugs in pediatric subpopulations, and to require manufacturers to develop appropriate drug formulations for each age group. Based on information from the FDA, CBO estimates that few such studies or new drug formulations for previously approved drugs would be required, and that the direct cost of those provisions would be less than $10 million annually.

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